the campaign for
SUSTAINABLE Rx PRICING

March 4, 2016

The Honorable Ron Wyden
Ranking Member
Senate Finance Committee
221 Dirksen Senate Building
Washington, D.C. 20510

The Honorable Charles Grassley
Senate Finance Committee
219 Dirksen Senate Building
Washington, D.C. 20510

Dear Senator Wyden and Senator Grassley:

Thank you for the opportunity to provide comment on ways Congress could address the important issue of high-cost prescription drugs. The Campaign for Sustainable Rx Pricing (the "Campaign") is a project of the National Coalition on Health Care Action Fund. Our members represent more than 80 organizations including consumers, hospitals, physicians, pharmacists, employers and health plans. The Campaign began its work in the spring of 2014, largely in response to the subject of your report - the unprecedented price tag that Gilead put on its prescription drug Solvadi. While launch prices and price increases for existing drugs have been a growing problem, this drug and its cost to the federal government, consumers, states, and employers represented a tipping point. The Campaign's mission is to foster and inform the debate on sustainable drug pricing and to develop market-based policy solutions that focus on transparency, competition and value.

The prescription drug market in the United States is broken. Brand name drug manufacturers operate in a fractured, demand-side market in which the doctors and patients do not have recourse for medication that is priced out of reach. Drug manufacturers essentially operate as monopolies and are often shielded from effective price competition through a lack of transparency about pricing, an absence of information about drug values, and decisions by manufacturers to put increased profits above access or affordability.

Many of the U.S. pharmaceutical market's problems can be traced to a lack of meaningful competition. Manufacturers with comparable brand name products have no real incentive to compete amongst themselves, instead pricing new products at higher and higher levels regardless of whether they represent an improvement over existing products.

Even more troubling, we are seeing a growing number of instances where manufacturers are setting and raising prices at previously unimaginable levels despite growing public outcry. When manufacturers see other companies succeed with this strategy, they claim that it is their legal obligation to shareholders to follow suit. The result is rapidly escalating health care costs, largely driven by unrelenting pharmaceutical price inflation.

As your investigation confirmed, we long suspected that Gilead priced Sovaldi with little regard for consumer affordability or access. Instead, they followed the example of other companies who

prioritize profit over patients and priced it at a level that they hoped would maximize revenue without too much backlash. We applaud your investigation for bringing this issue to the forefront of the Congressional debate and provide answers to the questions in your recent request for comment letter below.

What are the effects of a breakthrough, single source innovator drug on the marketplace?

There is no question that a breakthrough, single source innovator drug can have a significant impact on the marketplace. Unfortunately, many of these drugs come with astronomical prices. For example, Vertex priced two of its breakthrough therapies for Cystic Fibrosis, Kalydeco and Orkambi, at $311,000 and $259,000 respectively.[1] Notably, unlike Sovaldi which is a cure for Hepatitis C, these drugs must be taken every year. While only 10,500 patients will receive these treatments, the drug cost alone for these patients has been estimated to exceed $3 billion.[2] While a major breakthrough in treating Cystic Fibrosis patients, the staggering price tag for single source drugs is perpetuating this broken market and imposing an unsustainable burden on public and private payers. The impact on patients is even more troubling - this price tag has made this innovation effectively meaningless for those who cannot afford to use it.

In situations where breakthrough drugs have limited or no competition, policymakers should explore how expedited approval pathways, such as fast track designation, breakthrough therapy designation, accelerated approval, and priority review designation could be utilized to encourage additional market entrants and drive competition. Such a strategy could not only increase competition but will serve as an important protection to consumers if the first market entrant has to be withdrawn due to safety concerns.

However, competition is not always a magic bullet. We have seen troubling examples of other therapeutic classes where competing therapies have not brought down prices. Take, for example, drugs that treat multiple sclerosis. Several multiple sclerosis treatments have been around for decades. Basic economic theory says that new competing treatments should lead to a *decrease* in prices for the older products. Instead, the prices for old drugs have *increased* to match the prices of the new drugs, with some prices climbing by an average of 30 percent ***per year for two decades***, according to a report published in the journal *Neurology*.[3]

More transparency is needed to fully understand what is driving manufacturers' pricing decisions and develop solutions. This includes transparency for research and development costs and any factors used in setting the price, as well as clinical data. Please see our additional recommendations in the transparency section below.

[1] LaMattina, J. "Will The High Cost of Vertex's New Cystic Fibrosis Drug Push The U.S. To European Style Pricing?" *Forbes*, June 22, 2015.

[2] New York Times, "Orkambi, a New Cystic Fibrosis Drug, Wins F.D.A. Approval," July 2, 2015.

[3] D.M. Hartung, D.N. Bourdette, S.M. Ahmed, and R.H. Whitham, "The Cost of Multiple Sclerosis Drugs in the US and the Pharmaceutical Indiustry," *Neurology*, e-pub before print, April 24, 2015.

What role does the concept of "value" play in this debate, and how should an innovative therapy's value be represented in its price?

Investing in the development of information that assesses the effectiveness of different treatment options is a critical component to addressing the high price of prescription drugs. As the health care system drives to deliver higher value, there is insufficient evidence as to how new (and often expensive) drugs compare with older interventions. While other countries require data comparing various treatments to help reach a value-based price, the U.S. market allows drug manufacturers to set the price without asking manufacturers to justify the cost. The following efforts should be advanced to bring better information to providers and patients about the value of different treatments:

Expand Research on Treatment Effectiveness and Value: Consumers and providers should be empowered to know which treatments and drug regimens work and which are less effective. Policymakers should increase funding for private and public efforts aimed at providing information on the comparative effectiveness of different treatments to physicians and their patients which can help them make appropriate assessments about the value of different treatment approaches, particularly those with very high costs. A prime example is the Institute for Clinical and Economic Review (ICER). ICER is a non-profit organization that evaluates the evidence on the value of medical tests and treatments with an aim toward improving patient care and controlling costs. Recently, ICER released an important draft report on PCSK9 inhibitors for treatment of high cholesterol. In their draft assessment, ICER concluded that the price that best represents the overall benefits of these new drugs would be between $3,600 and $4,800 – a 67% discount off the manufacturers' list price of about $14,000 per year of treatment. Investments in the development of information such as this are critical for physicians, patients, and payers as more and more high-cost drugs are introduced into the health care system.

Drug Manufacturers Should be Required to Conduct Comparisons of New Products to Existing Products: Many other countries currently require drug manufacturers to provide a dossier of comparative effectiveness research (CER) studies to demonstrate that their product is better than the previously existing standard treatment. Expanding the availability and use of such research among US consumers, clinicians, and payers would help create evidence-based competition and reduce spending on unnecessary or ineffective treatments.

Government Programs Should Require Innovative Payment and Incentive Structures that Promote Value: Medicare should take the lead in supporting new payment models that promote value-based payments for drugs. While several models are starting to be developed in the private sector, including indication-based payments and outcomes-based contracts, government programs have lagged behind. These models can provide enhanced financial incentives for manufacturers of new drugs and medical technologies that are contingent upon agreed-upon standards for quality care and outcomes. Such strategies should be encouraged to the maximum extent possible.

What measures might improve price transparency for new higher-cost therapies while maintaining incentives for manufacturers to invest in new drug development?

Drug manufacturers regularly justify their pricing decisions by citing industry-funded research that claims that it costs $2.6 billion to bring a new drug to market.[4] Unfortunately, we have no way of verifying this often-disputed[5] figure. We also have no way of determining how much pharmaceutical companies actually invest in research and development activities. This missing information could play an extremely important role in assessing whether or not a drug's price is reasonable.

There is also a dearth of information regarding other factors that pharmaceutical companies consider in setting launch prices or in deciding to raise prices for drugs that are already on the market. Given the growing and significant impact pharmaceuticals have on overall health care spending, increased transparency is critical and cannot be tossed aside with general claims that any steps toward transparency will erode pharma investments in R&D.

Price Transparency Parity: As part of the drug approval process, manufacturers should be required to disclose information regarding the estimated unit price for the product, the cost of a course of treatment and a projection of federal spending on the product. This type of reporting could help prevent another Sovaldi-like situation where payers were surprised by unexpectedly high costs.[6] Subsequent to approval, manufacturers should be required to report, on an annual basis, any increase in the list price of that drug over a threshold as well as how many times a year the price of a drug has been increased. Information would be reported to HHS, with protections in place to exclude sensitive, proprietary information.

Transparency requirements are not without precedent. Currently, many entities in the health care sector report data to governmental entities. Health plan issuers are required to provide premium information to state insurance commissioners. In addition, issuers with rate increases above 10 percent are required to submit a justification to the government for review and must make summary information accessible to the public in an understandable format. As another example, hospitals, skilled nursing facilities, and certain other providers are required to submit cost report data to HHS annually. This includes information on facility characteristics, utilization data, costs and charges, and financial data. This policy would simply extend transparency to the pharmaceutical sector as well.

Ensuring a Better Return on Taxpayer Investments: While high prices are often justified based on the costs associated with research and development (R&D), there is virtually no public data showing that prices and development costs are linked. For example, the R&D for Sovaldi was largely conducted by a small bio-tech company that received the majority of its funding from the National Institutes of Health (NIH). Gilead purchased this bio-tech and recouped the cost of the acquisition in one year of sales of the $1,000 per pill drug. Manufacturers should be required to

[4] Tufts Center for the Study of Drug Development, "Cost to Develop and Win Marketing Approval for a New Drug Is $2.6 Billion," Press release, November 18, 2014,
[5] A. Carroll, "$2.6 Billion to Develop a Drug? New Estimate Makes Questionable Assumptions," *The Upshot, New York Times,* November 18, 2014.
[6] E. Pianin, "The $1,000 Pill That Could Cripple the VA's Budget," *The Fiscal Times,* October 8, 2014; J.L. Gonzalez, "Insurers Worry that $84,000 Hepatitis C Drug Sovaldi Could Break the Bank," *Newsweek,* May 28. 2014.

disclose research and development costs for drugs, including the portion of research funded by the manufacturer versus research funded by NIH; research by other academic entities; or research conducted by another pharmaceutical company (later acquired by the current manufacturer).

Leveraging Existing Laws that Protect Taxpayers: Existing law provides federal agencies with authority to license drugs to third-parties where the benefits of the product are not available to the public on reasonable terms. When products are funded (fully or partially) with NIH dollars, federal agencies should consider whether this authority could be utilized in cases where high prices or price increases threaten access to important medications, and they should inform Congress where they lack authority necessary to protect taxpayers.

Pricing Transparency Reports: A primary reason why the current "market" for drugs doesn't work is the lack of transparency surrounding drug pricing. Prices for drugs are clearly rising at rates that far exceed inflation and the level of any rebates or discounts offered by manufacturers. The federal government has data which can demonstrate how changes in list prices reflect changes in the prices paid by the Medicare and Medicaid programs. Under the Medicaid Rebate Program, pharmaceutical manufacturers are required to report certain key price terms to CMS for each of their drugs. These include Average Manufacturer Price ("AMP") and Best Price. While maintaining the confidentiality of this data, HHS could provide very useful analysis about drug prices based on the information it already collects from the Medicare and Medicaid programs. HHS should provide an annual report to the public which would include, among other items:

1. The top 50 price increases over the last year by a branded drug.
2. The top 50 price increases over the last year by a generic drug.
3. The top 50 drugs by annual spending and how much the government pays in total for these drugs.
4. Historical price increases for common drugs, including Medicare Part B drugs, over the most recent 10 year period.

Conclusion

The Coalition appreciates your consideration of these comments. If you have any questions, please do not hesitate to contact me (202-638-7151).

Sincerely,

John Rother
Executive Director, Campaign for Sustainable Rx Pricing
President and CEO, National Coalition on Health Care